PSY37

HOSPITAL-BASED RESOURCE UTILIZATION AMONG WEGENER'S GRANULOMATOSIS PATIENTS

Belk KW, Craver CW

MedAssets, Charlotte, NC, USA

OBJECTIVES: The purpose of this analysis is to examine hospital-based utilization in Wegener's granulomatosis (WG), a form of autoimmune disease characterized by necrotizing granulomas and vasculitis affecting approximately 3 out of every 100,000 people in the US. **METHODS:** A retrospective cross-sectional analysis examined inpatient (N=7,202) and outpatient visits (N=24,971) in the MedAssets health system data from 2009 to 2013. In addition to patient and hospital characteristics, the Charlson comorbidity index and its individual comorbidities were evaluated. Analysis of prevalent procedures and their comparative effects on utilization were explored. Measures of utilization included number of visits and length of stay (LOS). Negative-binomial multivariate regression was used to identify significant LOS drivers. **RESULTS:** Hospital utilization was primarily for outpatient services (77.6%) with a mean of 2.5 outpatient visits per patient. The primary diagnosis was WG for 58.6% of outpatients and16.6% of inpatients. Other common inpatient diagnoses included pneumonia (5.2%), septicemia (3.5%), and acute renal failure (3.4%). The sample included more females (54.6%) than males (45.3%) and 56.8% was between 40 and 69 years old. The mean Charlson score was 2.11 with renal disease (37.4%), chronic pulmonary disease (27.2%), diabetes (18.9%), congestive heart failure (11.1%), and rheumatic disease (10%) as the most prevalent comorbidities. The most common inpatient procedures were hemodialysis (26.3%), packed cell transfusions (18.0%), closed bronchial biopsy (8.1%), and closed renal biopsy (6.5%). Average LOS was 8.4 days. The most significant drivers associated with longer LOS included bronchial (IRR=1.72, p<.0001) and renal (IRR=1.48, p<.0001) biopsy procedures, packed cell transfusions (IRR=1.41 ,p<.0001), and therapeutic plasmapheresis (IRR=1.34, p<.0001). **CONCLUSIONS:** Although a rare disease WG patients consume significant amounts of health care resources in the hospital setting. Primary utilization occurs in the outpatient setting, however, when hospitalized LOS is often lengthy. Further research is required to understand the effect of interventions/treatments on mitigating progress of this disease.

SYSTEMIC DISORDERS/CONDITIONS – Patient-Reported Outcomes & Patient Preference Studies

PSY38

TREATMENT PATTERNS HIGHLIGHT UNMET NEEDS IN THE MANAGEMENT OF FIBROMYALGIA IN THE UNITED STATES

Yang M, Qian C

Daiichi Sankyo, Inc, Parsippany, NJ, USA

OBJECTIVES: Fibromyalgia is a disorder characterized by widespread chronic musculoskeletal pain. This study aims to understand the treatment patterns associated with currently available and commonly used medications in the management of fibromyalgia. **METHODS:** This retrospective study used the MarketScan claim database to identify patients who had a first fibromyalgia diagnosis code (ICD-9-CM: 729.1) in 2009-2011 with a repeat diagnosis within a year; were at least 18 years old; and had continuous enrollment for ≥12 months before and after the date of first diagnosis, i.e. the index date. The pain treatments assessed were: anticonvulsants (pregabalin, gabapentin), antidepressants (amitriptyline, cyclobenzaprine, venlafaxine, duloxetine, milnacipran), and opioid (tramadol). Adherence was evaluated using percent of days covered (PDC) and categorized into low (<50%), medium (50%-80%), and high (≥80%). The 1-year discontinuation (defined as the first drug supply gap ≥90 days) rate was estimated using Kaplan-Meier curve. **RESULTS:** A total of 240,144 patients met the inclusion criteria; 68% were female and the study sample mean (SD) age was 48 (13.8) years. Only 31% (N=74,738) of the patients initiated a treatment of interest within a year after the index date. The 3 most frequently dispensed ones were cyclobenaprine (27.4%), tramadol (17.9%), and gabapentin (16.3%). Duloxetine, pregabalin, and milnacipran accounted for 13.6%, 8.9%, and 3.8% of treated patients respectively. Adherence was suboptimal for all of these treatments: mean PDCs (% with high adherence) were 59% (39%), 47% (24%), 44% (22%), 43% (21%), 27% (9%), and 20% (5%) for duloxetine, pregabalin, gabapentin, milnacipran, tramadol, and cyclobenaprine, respectively; and the corresponding 1-year discontinuation rates were 52%, 65%, 67%, 72%, 80%, and 90%, respectively. **CONCLUSIONS:** The majority of patients diagnosed with fibromyalgia were not treated with the assessed therapies in this study cohort. Adherence and persistence with the current pain medications were sub-optimal.

PSY39

ADHERENCE TO PROPHYLACTIC TREATMENT IN HEMOPHILIA AS MEASURED USING THE VERITAS-PRO AND ANNUAL BLEED RATE (ABR)

Duncan NA[1], Kronenberger WG[2], Krishnan S[3], Shapiro AD[1]

[1]*Indiana Hemophilia and Thrombosis Center, Indianapolis, IN, USA,* [2]*Indiana University, Indianapolis, IN, USA,* [3]*Biogen Idec, Cambridge, MA, USA*

OBJECTIVES: Few studies exist to support the hypothesis that nonadherence to prophylactic treatment of hemophilia leads to increased bleeding. The VERITAS-Pro, a validated measure of adherence to prophylaxis, allows objective measurement of the relationship between adherence and patient outcomes. The study objective was to correlate VERITAS-Pro scores and annual bleed rate (ABR), defined as the number of bleeds a patient has in a given year. **METHODS:** The study sample was comprised of patients utilizing prophylaxis treatment who completed the VERITAS-Pro for the validation study published by Duncan et al in Haemophilia in 2007 (n=66). ABR was extrapolated from patient infusion logs and, for those patients with incomplete or missing logs, from electronic medical records. Pearson and Spearman correlational analyses were run between VERITAS-Pro scores and ABR. A cutoff analysis was done in which the 70th percentile was chosen as a clinically useful cutoff score and VERITAS-Pro scores were dichotomized and coded as 0 (good adherence) if below the 70th percentile and 1 (poor adherence) if above the 70th percentile. ABR was also dichotomized into no bleeds (0 ABR), the true goal of prophylaxis, versus 1 or more bleeds (1+ ABR). **RESULTS:** Reported ABR ranged from 0 to 54 (median 1). There was not a significant relationship between VERITAS-Pro score and ABR. There was a significantly greater percentage of patients experiencing one or more bleeds in the VERITAS-Pro poor adherence group (86%) than in the VERITAS-Pro good adherence group (62%). **CONCLUSIONS:** VERITAS-Pro scores reflecting adherence are significant predictors of ABR; individuals with poor adherence on the VERITAS-Pro are more likely to have at least 1 bleed per year versus those with good adherence. However, the high percentage of patients experiencing one or more bleeds in both groups indicates that factors other than adherence may impact annualized bleed rates.

PSY40

INDIRECT COMPARISON OF THE EFFICACY OF RECOMBINANT FACTOR VIII FC FUSION PROTEIN AND OTHER FACTOR VIII PRODUCTS FOR PROPHYLAXISMODELING THE EFFECT OF COMPLIANCE

Iorio A[1], Krishnan S[2], Huynh L[3], Karner P[3], Duh MS[3], Yermakov S[3]

[1]*McMaster University, Hamilton, ON, Canada,* [2]*Biogen Idec, Cambridge, MA, USA,* [3]*Analysis Group, Boston, MA, USA*

OBJECTIVES: For people with hemophilia A, factor VIII (FVIII) prophylaxis is burdensome, potentially leading to poor compliance. Treatment adherence and outcomes may be improved with drugs requiring less frequent infusions. In the absence of head-to-head direct comparative evidence from clinical trials, this analysis indirectly compared the prophylactic efficacy of recombinant FVIII Fc fusion protein (rFVIIIFc) with the published results of current rFVIII products and simulated effects of potential differences in real-world compliance between regimens. **METHODS:** rFVIIIFc and rFVIII were indirectly compared using data from previously treated subjects in the A-LONG phase 3 study (rFVIIIFc; individualized arm) and published clinical studies of routine prophylaxis (rFVIII; identified by literature search). Efficacy was compared using reported differences in mean annualized bleed rates (ABRs) for individual and pooled results using meta-analysis with random effects. Unreported standard deviations of ABR were estimated assuming a Poisson distribution and adjusted for over-dispersion. A model was developed to assess the effect of compliance changes on ABR. **RESULTS:** This analysis included published results from the A-LONG study (severe hemophilia; rFVIIIFc; Mahlangu 2013) and 4 studies of rFVIII (moderate/severe hemophilia; Advate®: Tarantino 2004, Shapiro 2003, and Valentino 2012; Xyntha®: Recht 2009). Infusion frequencies were 1.4-2.4 (median 2.0) times/week for rFVIIIFc and 2.3-4 times/week for rFVIII. Mean ABR for rFVIIIFc was 2.9; the pooled mean ABR estimate for rFVIII was 4.8 (I^2 =44.2%, ΔABR=1.8; P=0.003). Simulations showed that statistically significant improvements in mean ABR would result from improving compliance with rFVIIIFc by ≥6-12 percentage points. CONCLUSIONSResults of this unadjusted indirect comparison of clinical studies suggest that routine prophylaxis with rFVIIIFc may result in a lower mean ABR than that of other rFVIII products examined. Moreover, potential improvements in compliance associated with less burdensome dosing requirements, as suggested by studies in other chronic diseases, may result in better effectiveness with rFVIIIFc.

PSY41

INDIRECT COMPARISON OF THE EFFICACY OF RECOMBINANT FACTOR IX FC FUSION PROTEIN AND OTHER FACTOR IX PRODUCTS FOR PROPHYLAXIS: SIMULATING THE EFFECT OF COMPLIANCE ON REAL-WORLD EFFECTIVENESS

Iorio A[1], Krishnan S[2], Huynh L[3], Karner P[3], Duh MS[3], Yermakov S[3]

[1]*McMaster University, Hamilton, ON, Canada,* [2]*Biogen Idec, Cambridge, MA, USA,* [3]*Analysis Group, Boston, MA, USA*

OBJECTIVES: Hemophilia B prophylaxis with factor IX (FIX) requires frequent infusions, potentially leading to poor compliance and reduced therapeutic effectiveness. In the absence of head-to-head direct comparative evidence from clinical trials, this analysis indirectly compared the prophylactic efficacy of recombinant FIX Fc fusion protein (rFIXFc) and other rFIX products, which require more frequent infusions. Additionally, we simulated the effects of potential differences in real-world compliance between regimens. **METHODS:** rFIXFc and rFIX were indirectly compared using data from clinical trials of previously treated subjects administered rFIXFc (B-LONG phase 3 study, weekly prophylaxis arm) or rFIX (published clinical studies of routine prophylaxis identified by literature search). Efficacy was compared using reported differences in mean annualized bleed rates (ABRs) for individual and pooled results using meta-analysis with random effects. Unreported standard deviations of ABR were estimated assuming a Poisson distribution and adjusted for over-dispersion. A model simulating the effect of improved compliance on ABR was developed. **RESULTS:** This analysis included results from the published B-LONG study (severe hemophilia; rFIXFc; Powell 2013) and 4 published studies of rFIX (moderate/severe hemophilia; BeneFIX®: Roth 2001, Lambert 2007, and Korth-Bradley 2011; Rixubis®: Windyga 2012). Infusion frequencies were once weekly for rFIXFc, and 1 to >3 times/week (Roth 2001, Lambert 2007) or 1-2 times/week (Korth-Bradley 2011, Windyga 2012) for rFIX. Mean ABR for rFIXFc was 3.07; the pooled mean ABR estimate for rFIX was 3.84 based on these clinical studies (I^2=57.5%, ΔABR=0.77; P=0.23). Simulations showed that statistically significant improvements in mean ABR would result from improving compliance with rFIXFc by ≥9-14 percentage points. **CONCLUSIONS:** Based on simulations of potential differences in real-world compliance, less burdensome dosing with rFIXFc may lead to improved real-world effectiveness, consistent with findings in other chronic diseases.

PSY42

UNDERSTANDING WHAT PATIENTS VALUE AND THEIR WILLINGNESS-TO-PAY (WTP) FOR HEMOPHILIA THERAPIESA DISCRETE CHOICE EXPERIMENT

Chaugule S[1], Hay JW[1], Young G[2]

[1]*University of Southern California, Los Angeles, CA, USA,* [2]*Keck School of Medicine, USC, Los Angeles, CA, USA*